EMA/216880/2019

European Medicines Agency decision

P/0166/2019

of 15 May 2019

on the acceptance of a modification of an agreed paediatric investigation plan for Fc- and CDR-modified humanised monoclonal antibody against C5 (EMEA-002077-PIP01-16-M02) in accordance with Regulation (EC) No 1901/2006 of the European Parliament and of the Council

Disclaimer

This decision does not constitute entitlement to the rewards and incentives referred to in Title V of Regulation (EC) No 1901/2006.

Only the English text is authentic.

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The European Medicines Agency,

Having regard to the Treaty on the Functioning of the European Union,

Having regard to Regulation (EC) No 1901/2006 of the European Parliament and of the Council of 12 December 2006 on medicinal products for paediatric use and amending Regulation (EEC) No. 1768/92, Directive 2001/20/EC, Directive 2001/83/EC and Regulation (EC) No 726/2004[1],

Having regard to Regulation (EC) No 726/2004 of the European Parliament and of the Council of 31 March 2004 laying down Community procedures for the authorisation and supervision of medicinal products for human and veterinary use and establishing a European Medicines Agency[2],

Having regard to the European Medicines Agency's decision P/0199/2017 issued on 14 July 2017 and the decision P/0356/2017 issued on 1 December 2017,

Having regard to the application submitted by Alexion Europe SAS on 20 December 2018 under Article 22 of Regulation (EC) No 1901/2006 proposing changes to the agreed paediatric investigation plan with a deferral,

Having regard to the opinion of the Paediatric Committee of the European Medicines Agency, issued on 29 March 2019, in accordance with Article 22 of Regulation (EC) No 1901/2006,

Having regard to Article 25 of Regulation (EC) No 1901/2006,

Whereas:

(1) The Paediatric Committee of the European Medicines Agency has given an opinion on the acceptance of changes to the agreed paediatric investigation plan.

(2) It is therefore appropriate to adopt a decision on the acceptance of changes to the agreed paediatric investigation plan.

[1] OJ L 378, 27.12.2006, p.1.
[2] OJ L 136, 30.4.2004, p. 1.

Has adopted this decision:

Article 1

Changes to the agreed paediatric investigation plan for Fc- and CDR-modified humanised monoclonal antibody against C5, concentrate for solution for infusion, intravenous use, are hereby accepted in the scope set out in the opinion of the Paediatric Committee of the European Medicines Agency annexed hereto, together with its appendices.

Article 2

This decision is addressed to Alexion Europe SAS, 1-15 Avenue Edouard Belin, 92500 - Rueil-Malmaison, France.

EMA/916181/2019
Amsterdam, 29 March 2019

Opinion of the Paediatric Committee on the acceptance of a modification of an agreed Paediatric Investigation Plan

EMEA-002077-PIP01-16-M02

Scope of the application

Active substance(s):

Fc- and CDR-modified humanised monoclonal antibody against C5

Condition(s):

Treatment of paroxysmal nocturnal haemoglobinuria

Pharmaceutical form(s):

Concentrate for solution for infusion

Route(s) of administration:

Intravenous use

Name/corporate name of the PIP applicant:

Alexion Europe SAS

Basis for opinion

Pursuant to Article 22 of Regulation (EC) No 1901/2006 as amended, Alexion Europe SAS submitted to the European Medicines Agency on 20 December 2018 an application for modification of the agreed paediatric investigation plan with a deferral as set out in the European Medicines Agency's decision P/0199/2017 issued on 14 July 2017 and the decision P/0356/2017 issued on 1 December 2017.

The application for modification proposed changes to the agreed paediatric investigation plan.

The procedure started on 29 January 2019.

Scope of the modification

Some measures of the Paediatric Investigation Plan have been modified.

Opinion

1. The Paediatric Committee, having assessed the application in accordance with Article 22 of Regulation (EC) No 1901/2006 as amended, recommends as set out in the appended summary report:

   - to agree to changes to the paediatric investigation plan in the scope set out in the Annex I of this opinion.

   The Norwegian Paediatric Committee member agrees with the above-mentioned recommendation of the Paediatric Committee.

2. The measures and timelines of the paediatric investigation plan are set out in the Annex I.

This opinion is forwarded to the applicant and the Executive Director of the European Medicines Agency, together with its annex and appendix.

Annex I

The subset(s) of the paediatric population and condition(s) covered by the waiver and the measures and timelines of the agreed paediatric investigation plan (PIP)

1. Waiver

Not applicable

2. Paediatric investigation plan

2.1. Condition:

Treatment of Paroxysmal Nocturnal Haemoglobinuria

2.1.1. Indication(s) targeted by the PIP

Treatment of Paroxysmal Nocturnal Haemoglobinuria

2.1.2. Subset(s) of the paediatric population concerned by the paediatric development

From birth to less than 18 years of age

2.1.3. Pharmaceutical form(s)

Concentrate for solution for infusion

2.1.4. Measures

Area	**Number of measures**	**Description**
Quality-related studies	0	Not applicable
Non-clinical studies	0	Not applicable
Clinical studies	1	**Study 1** Open-label, multicentre, single arm trial to evaluate pharmacokinetic (PK) and pharmacodynamic (PD) parameters, efficacy and safety of Fc- and CDR-modified humanised monoclonal antibody against C5 (ALXN1210) in children with Paroxysmal Nocturnal Haemoglobinuria (PNH)
Extrapolation, modelling and simulation studies	3	**Study 2** Modelling and simulation dose-finding study **Study 3** Extrapolation study to evaluate the efficacy, PK/PD and safety of ALXN1210 in paediatric PNH patients from 12 to less than 18 years of age

		Study 4 Extrapolation study to evaluate the efficacy, PK/PD and safety of ALXN1210 in paediatric PNH patients from birth to less than 12 years of age
Other studies	0	Not applicable
Other measures	0	Not applicable

3. Follow-up, completion and deferral of PIP

Concerns on potential long term safety/efficacy issues in relation to paediatric use:	Yes
Date of completion of the paediatric investigation plan:	By December 2021
Deferral for one or more measures contained in the paediatric investigation plan:	Yes